

Fig. 1a

Non-Transduced hMSCs in Immuno-competent

mouse muscle

Day 3

DiI + hMSCs

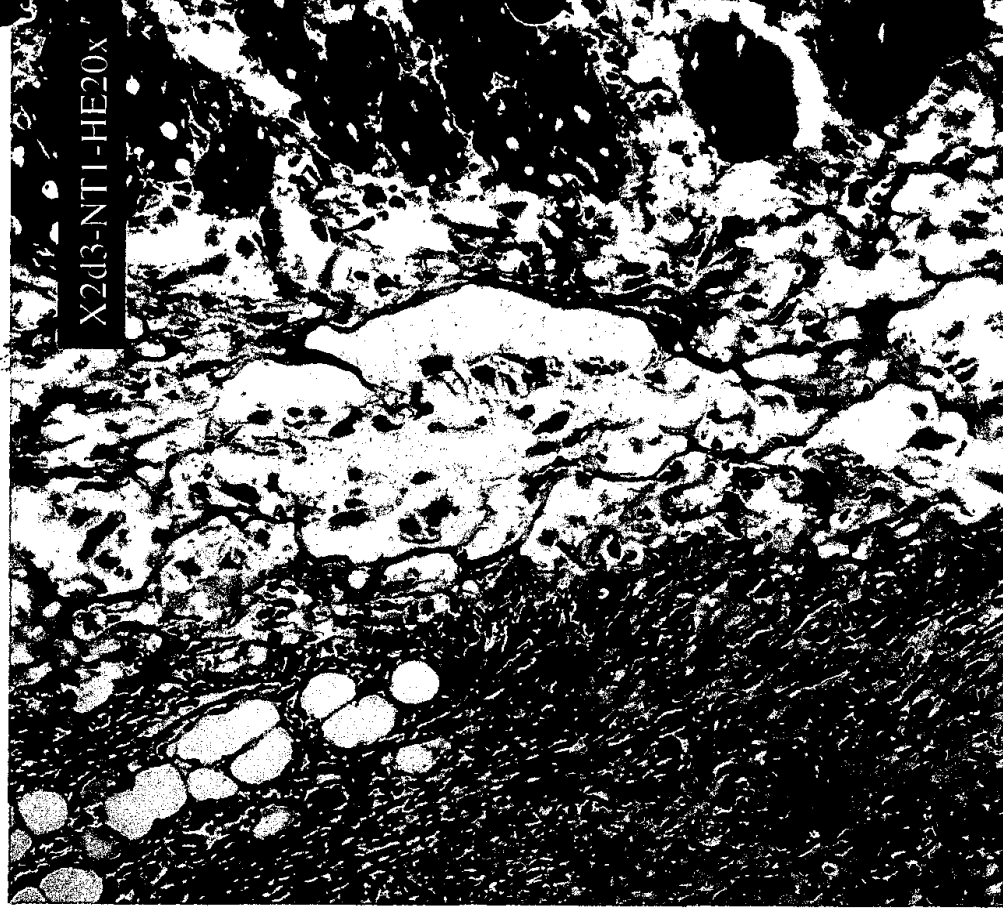
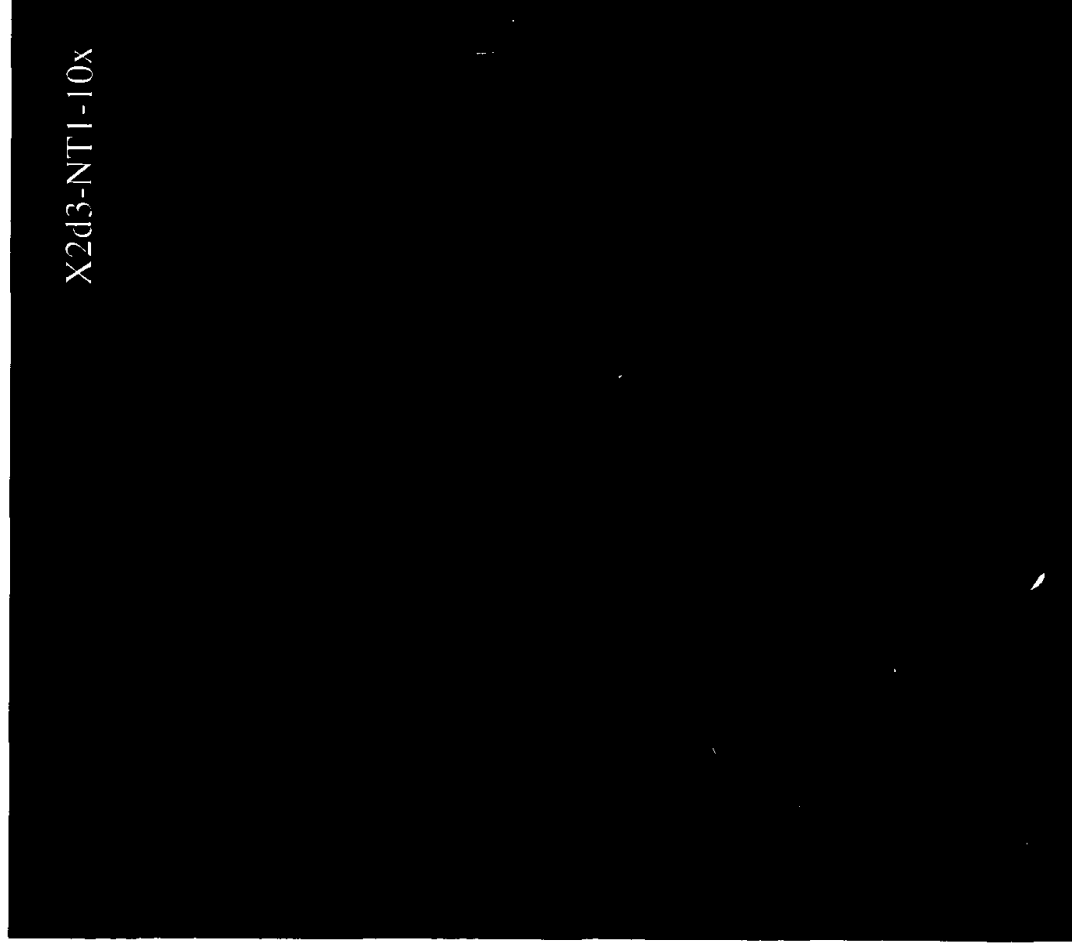


Fig.1b

Human MSCs in Mouse Muscle: Day-7

(H&E)



DiI labelled cells +
Sybr-green staining
of all nuclei

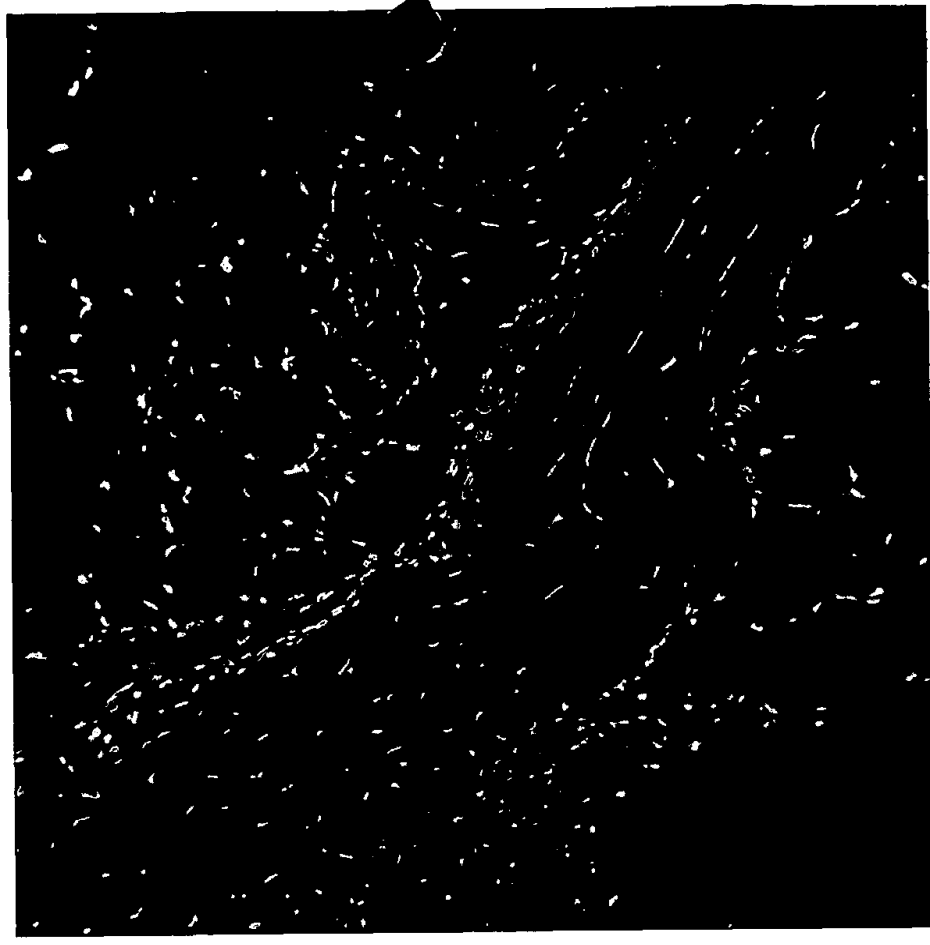


Fig.1c Human MSCs in Mouse Muscle: Day-7

(H&E, 20X)

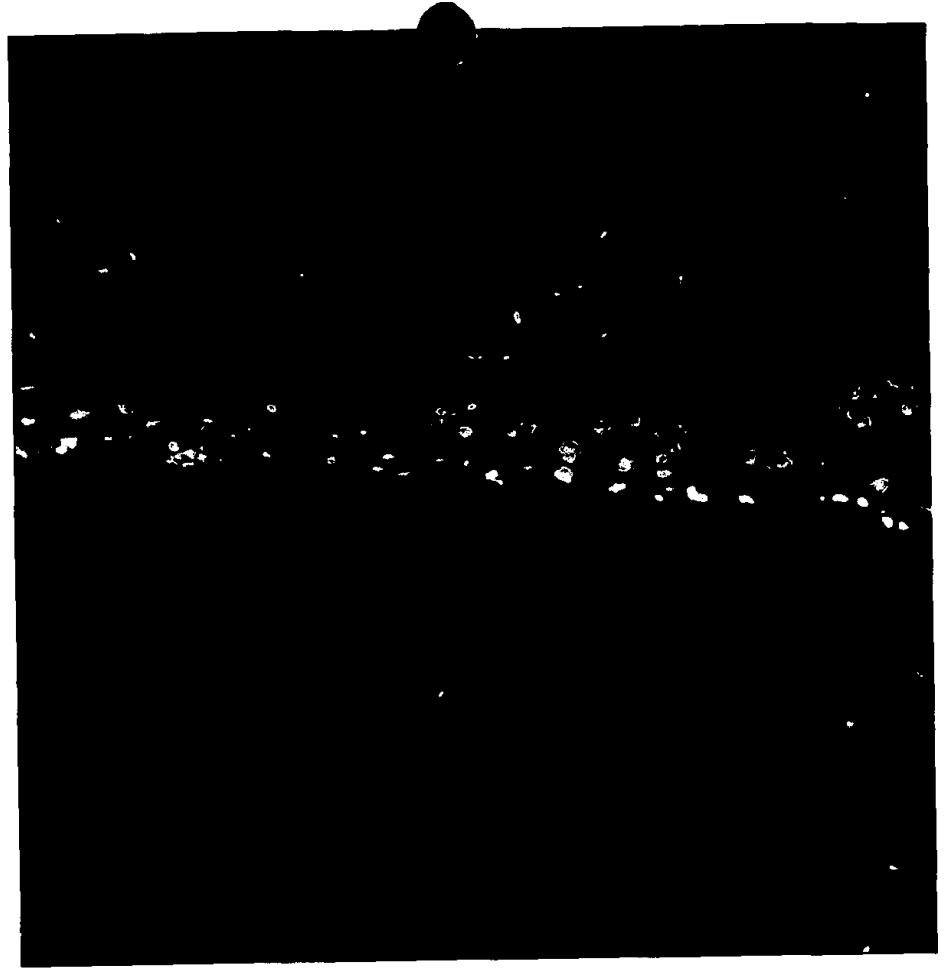


Fig.1d Human MSCs in Mouse Muscle: Day 14

Fluorescent Field, 10X



H&E 40X



**Fig. 1e DiI-Positive α -Gala Transduced hMSCs in
Immuno-competent mouse muscle**

d29



d43

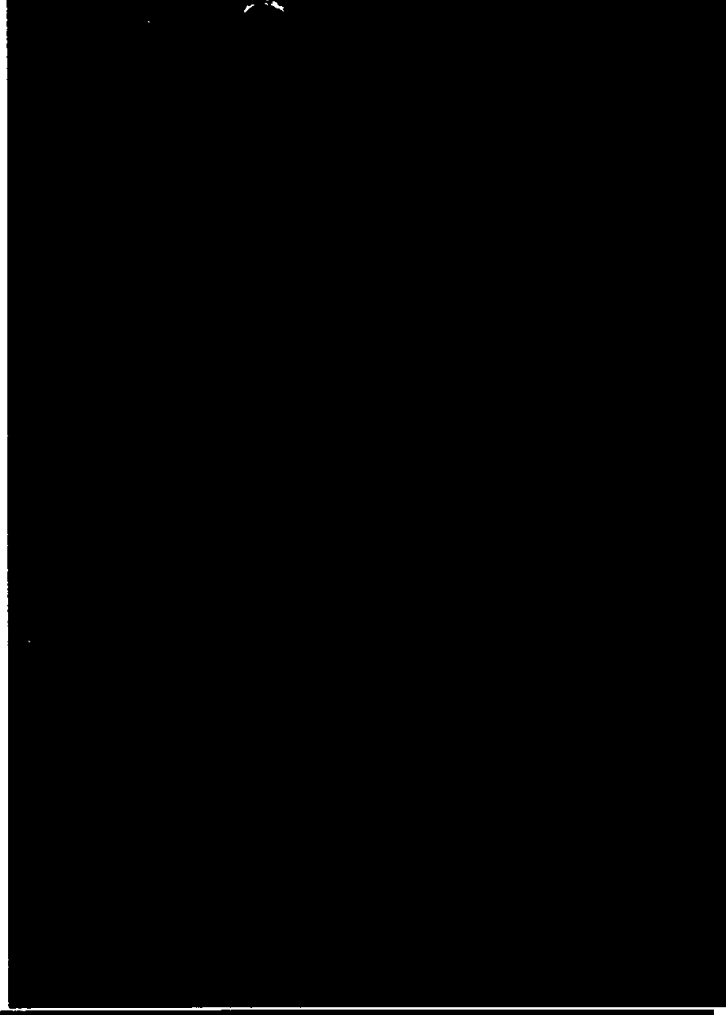


Fig.1f

Human α GalA-Transduced MSCs in Mouse Muscle: Day-57

10X

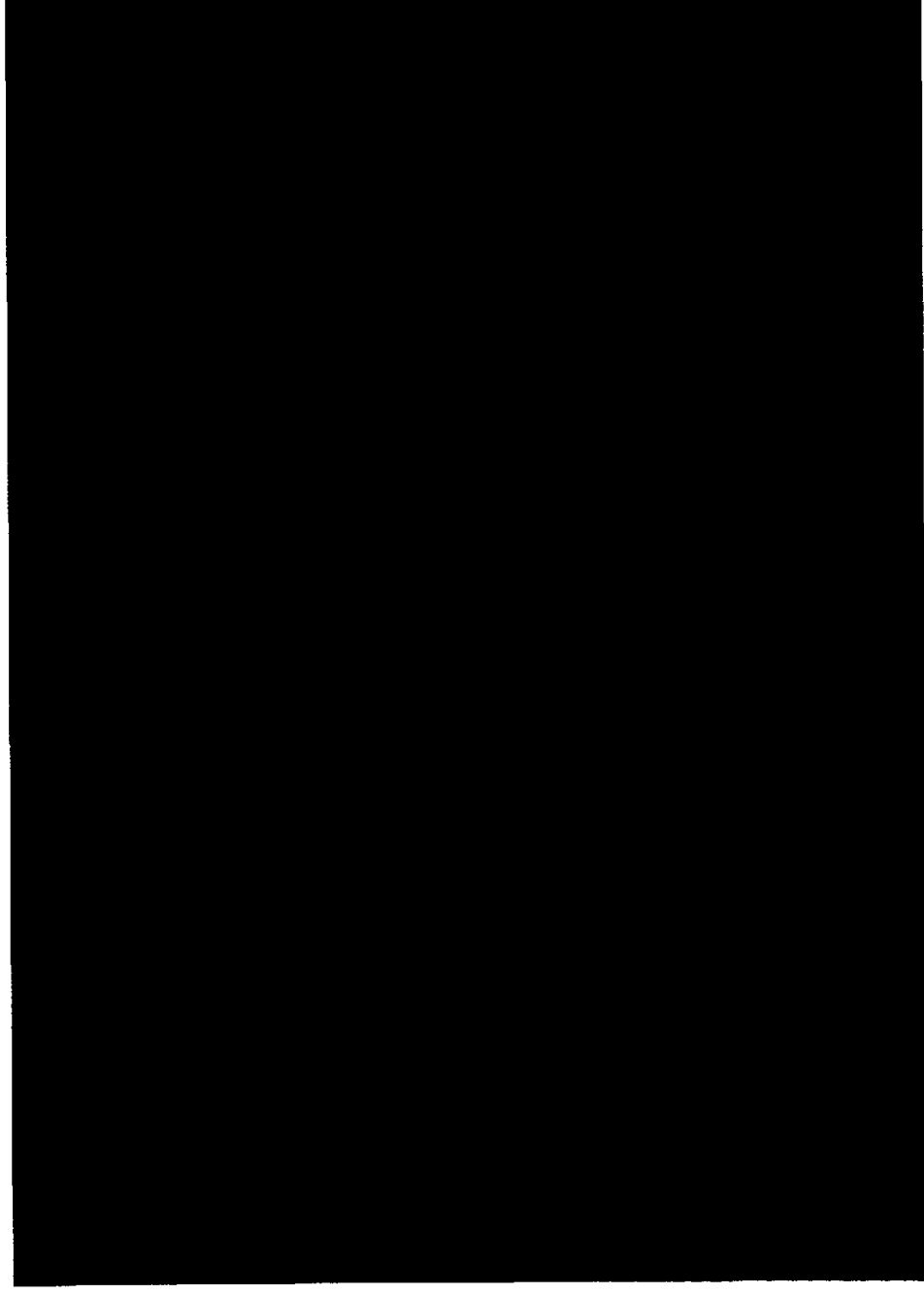


Figure-2

D3: IM injection of DiI-labeled PBMCs

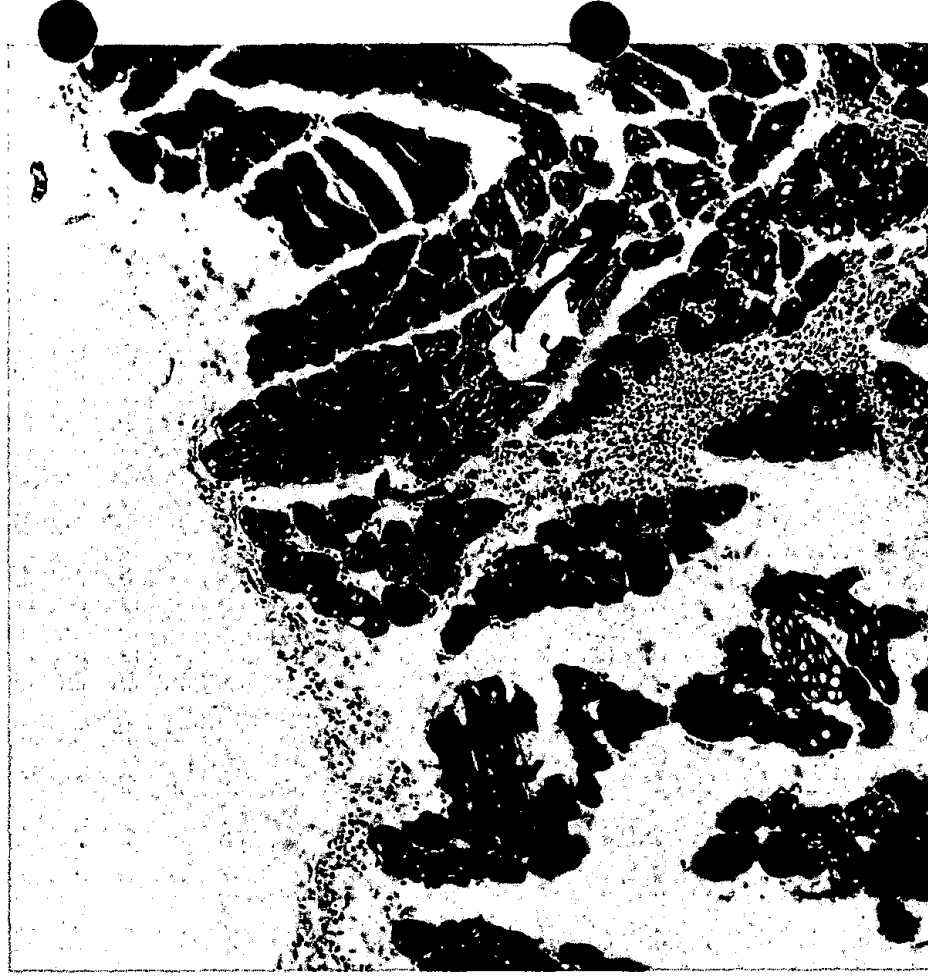
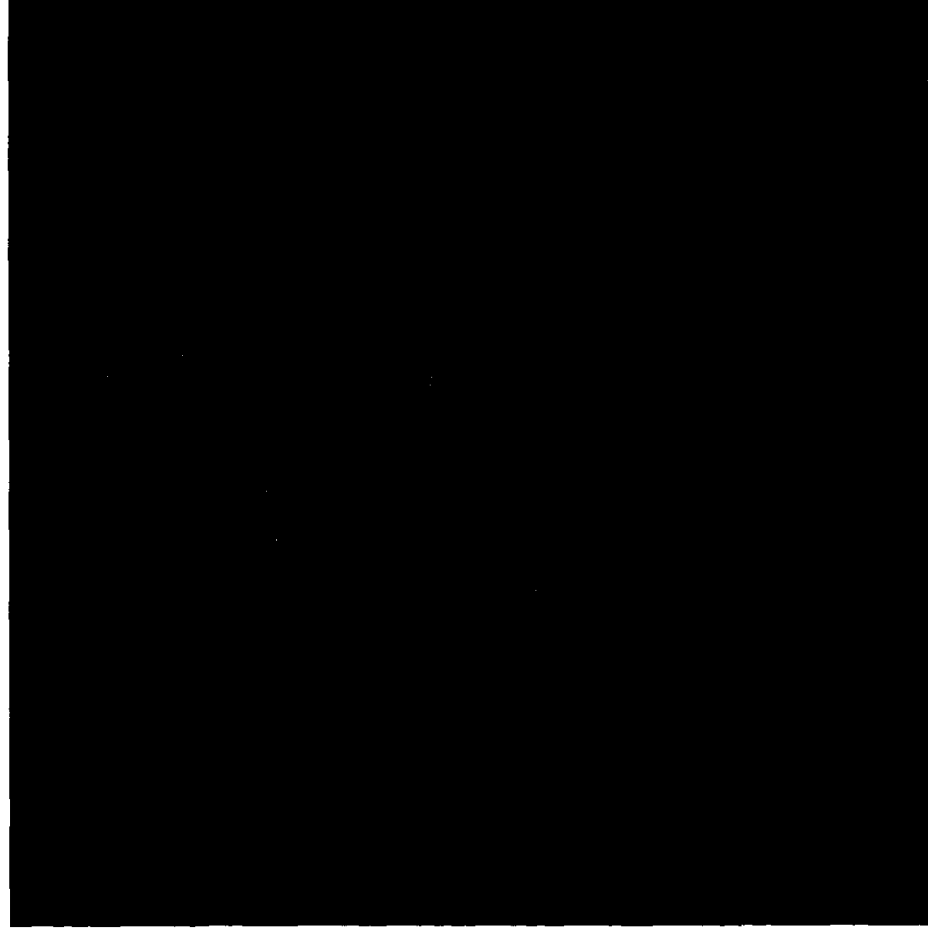
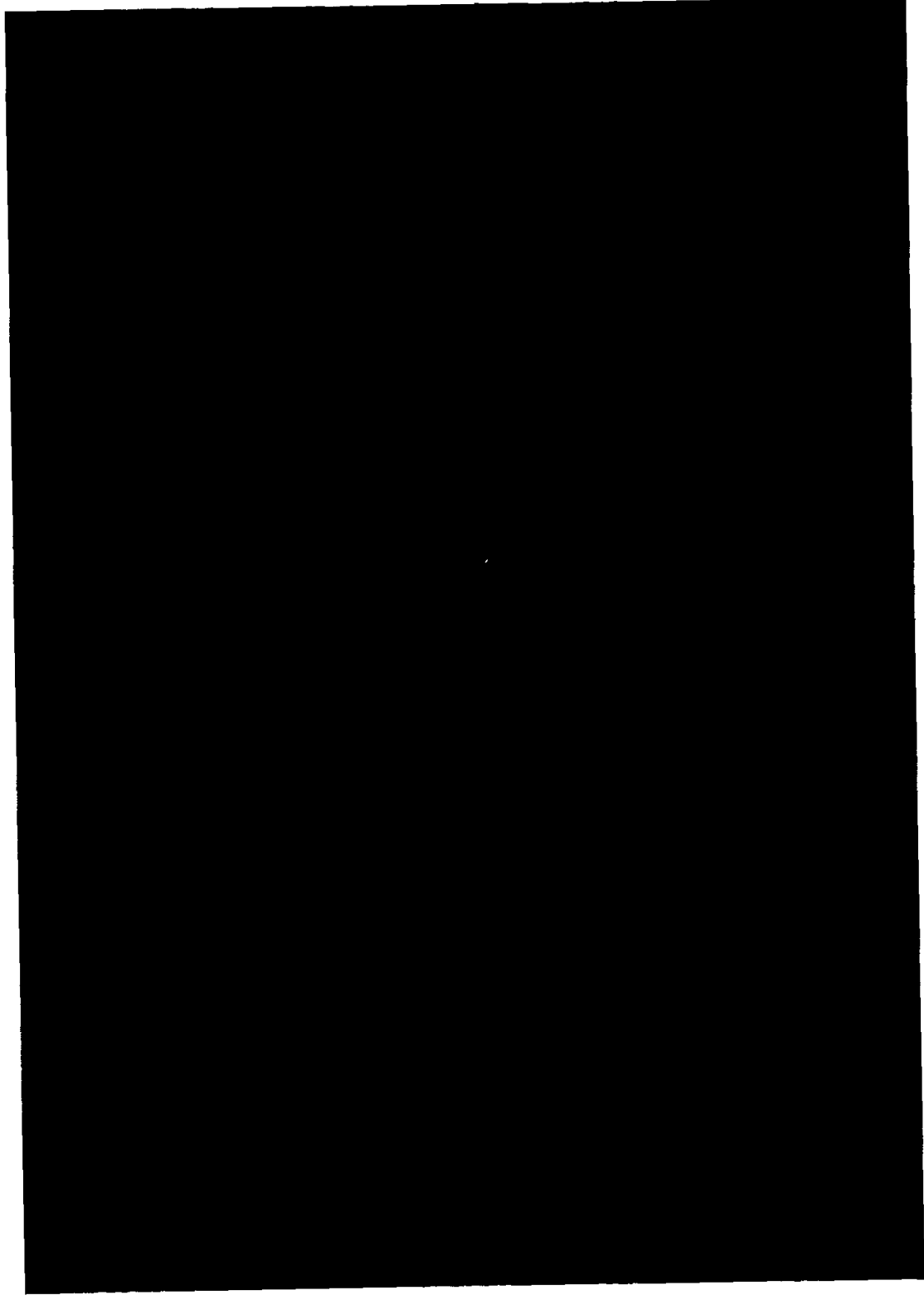


Figure-3

D3 IM injection of saline



Fig. 4 **6-week DAPI labeled hMSCs in Mouse Muscle**



Red and Blue Florescence Channels merged

Fig.5 α GalA Immunostain of Muscle from Mouse injected IM with α GalA-hMSCs: 14 days

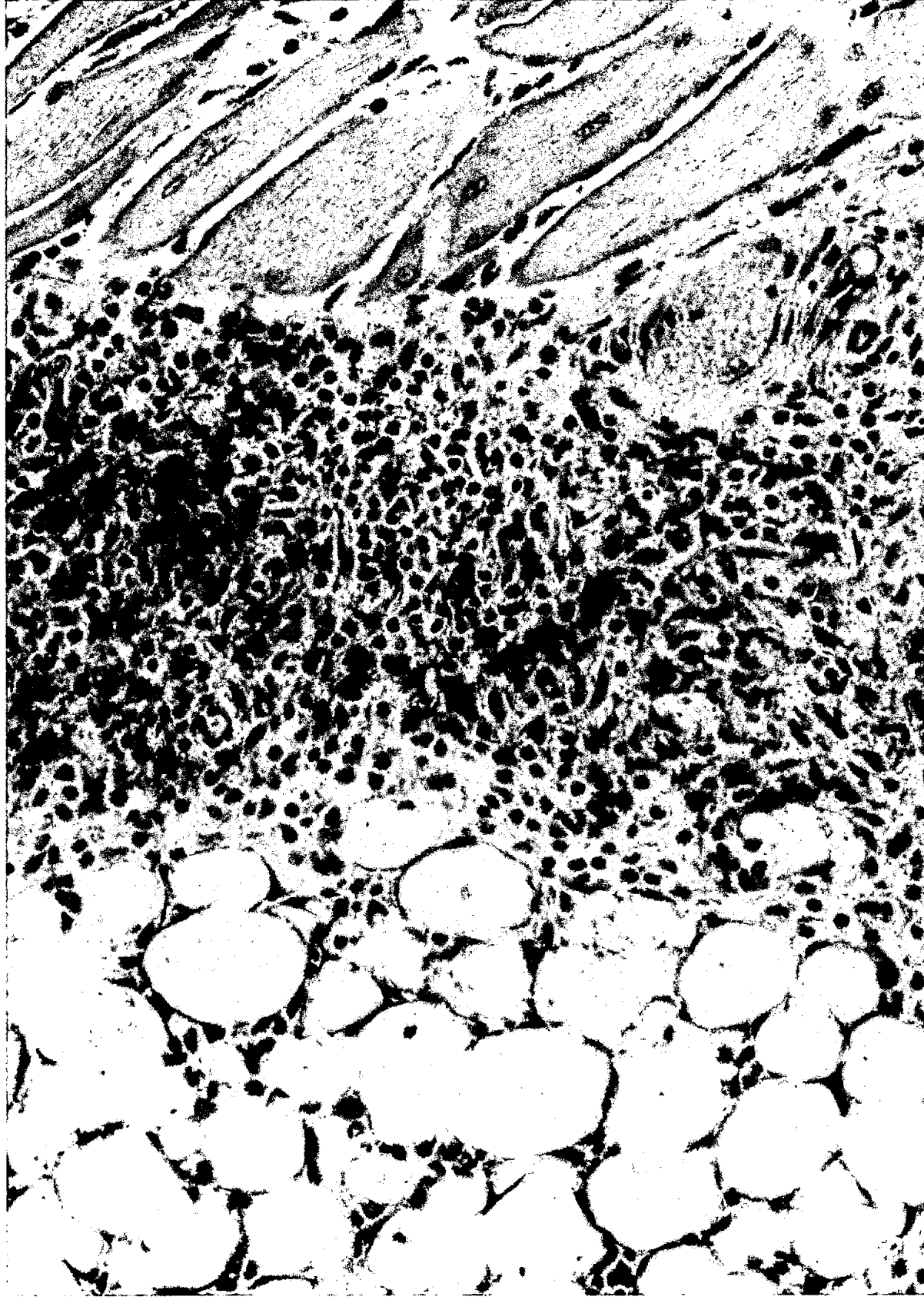
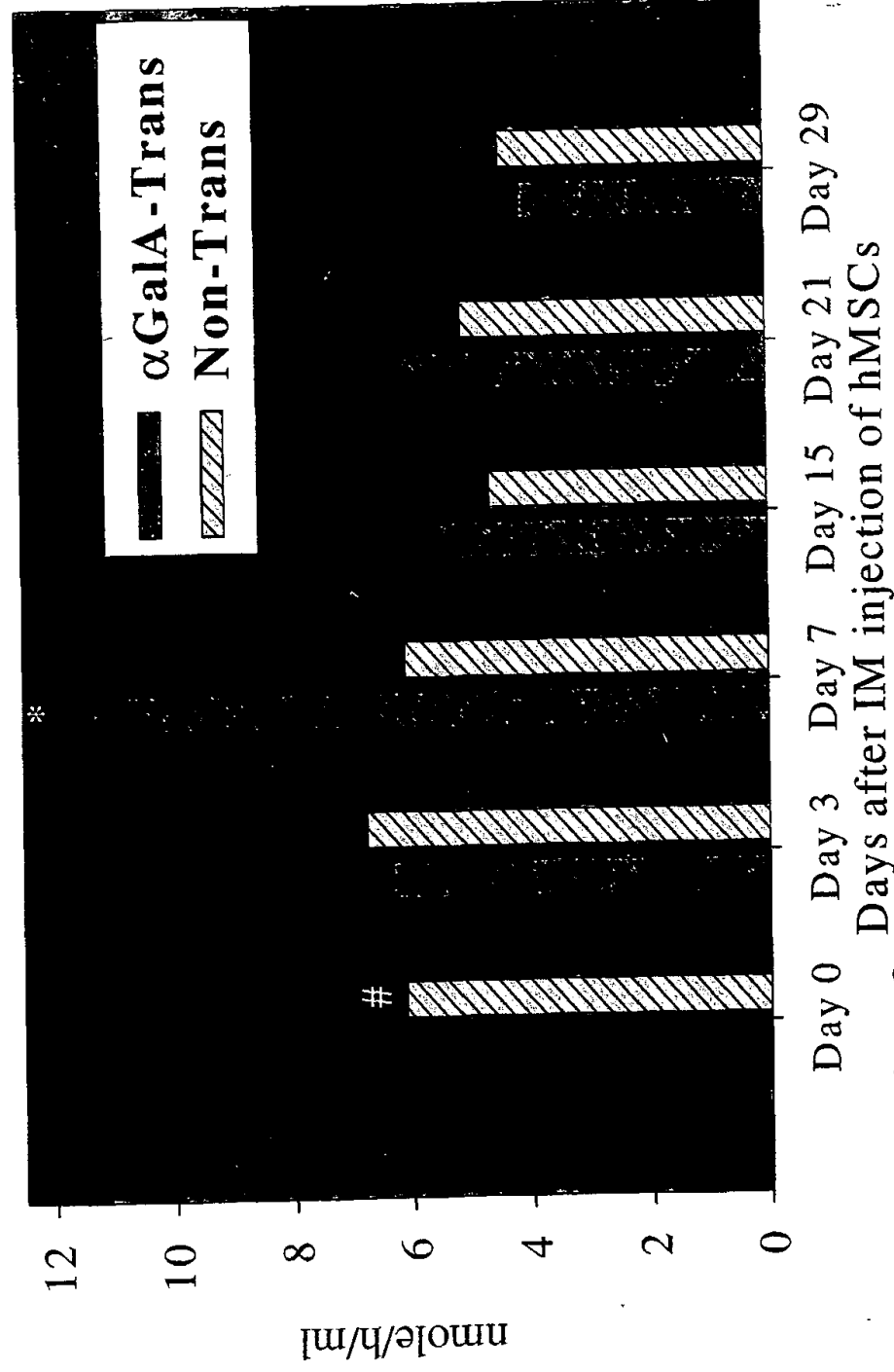


Figure 6

α -GAL activity in Plasma of Mice IM-injected with α -GAL-Transduced hMSCs

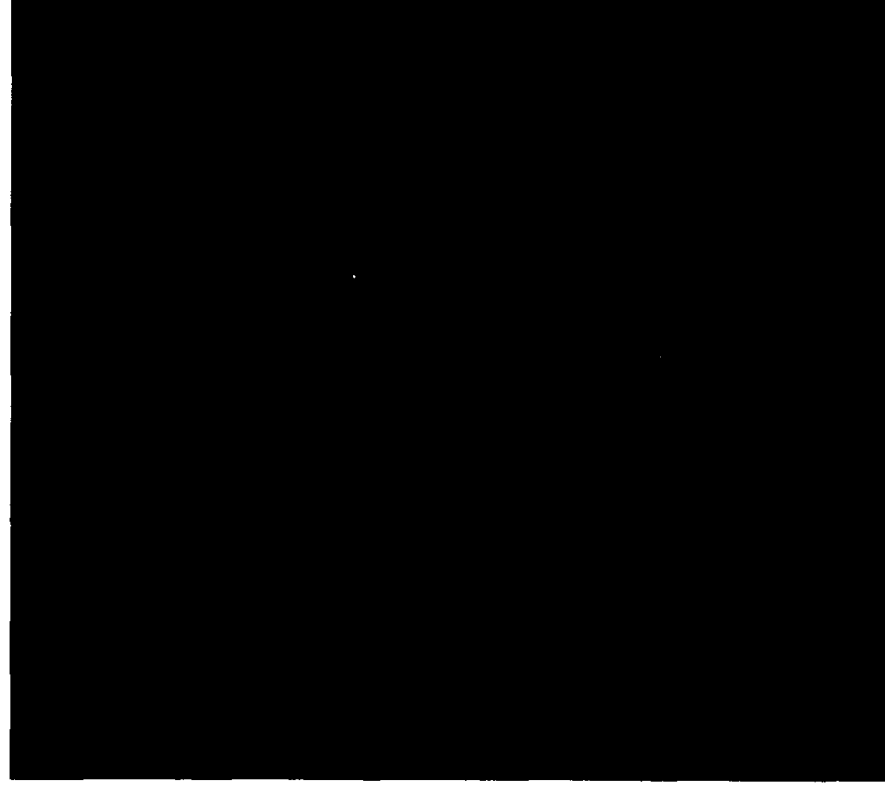


#: No MSCs, * n=2,

Fabry Mice plasma: 0.5 nmole/h/ml, PNAS 97:7515

Human-specific Alu sequence staining of hMSCs injected into Immuno-competent mouse muscle

Dil-positive cells

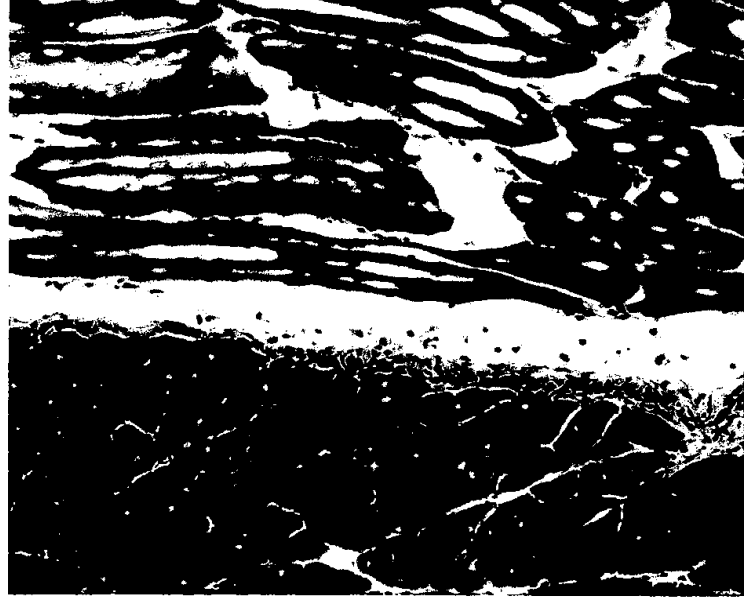


Alu-sequence positive cells

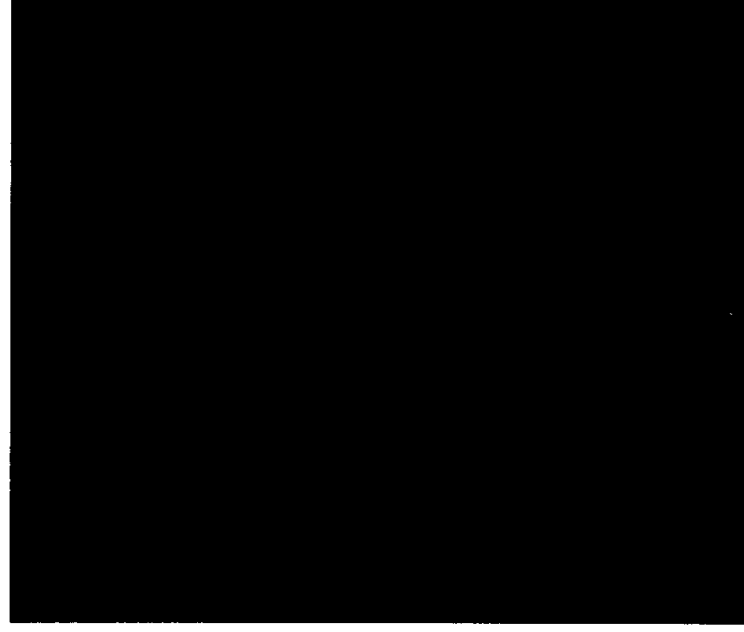


Human MSCs in Mouse Muscle: 43 days

H & E



Dil Positive Cells



Alu-Positive cells



Figure 7c

Day 57 DiI/Alu

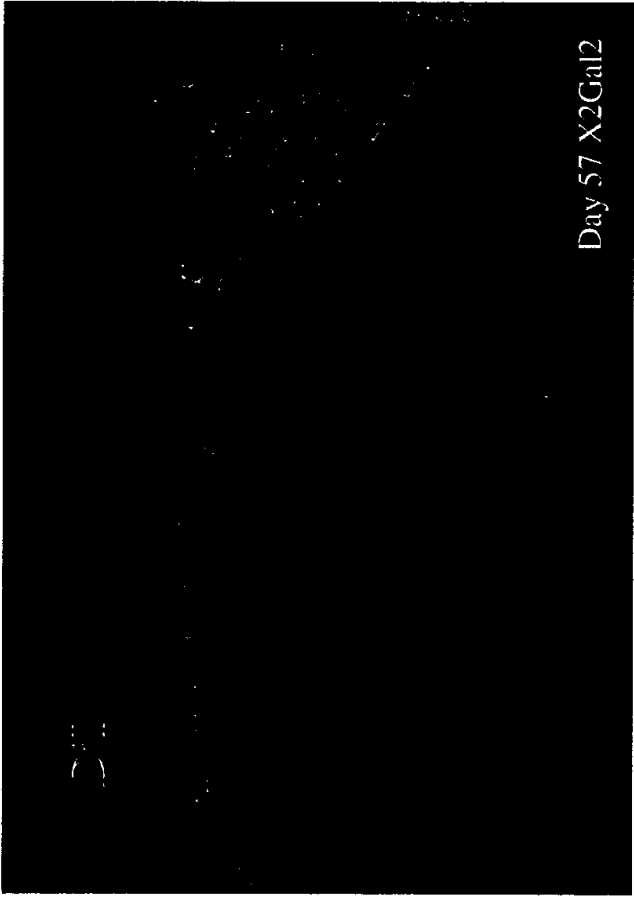
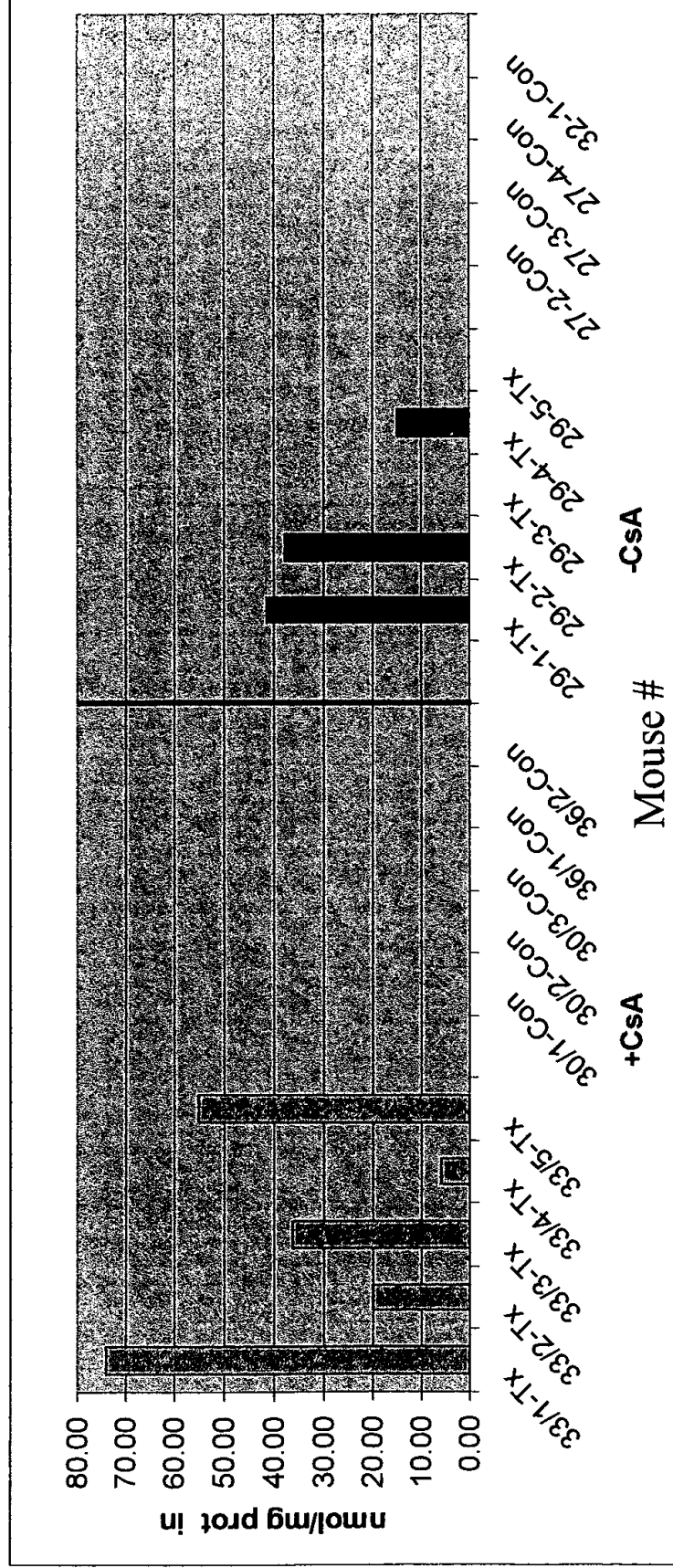


Figure-8a

FOHFF 6226660

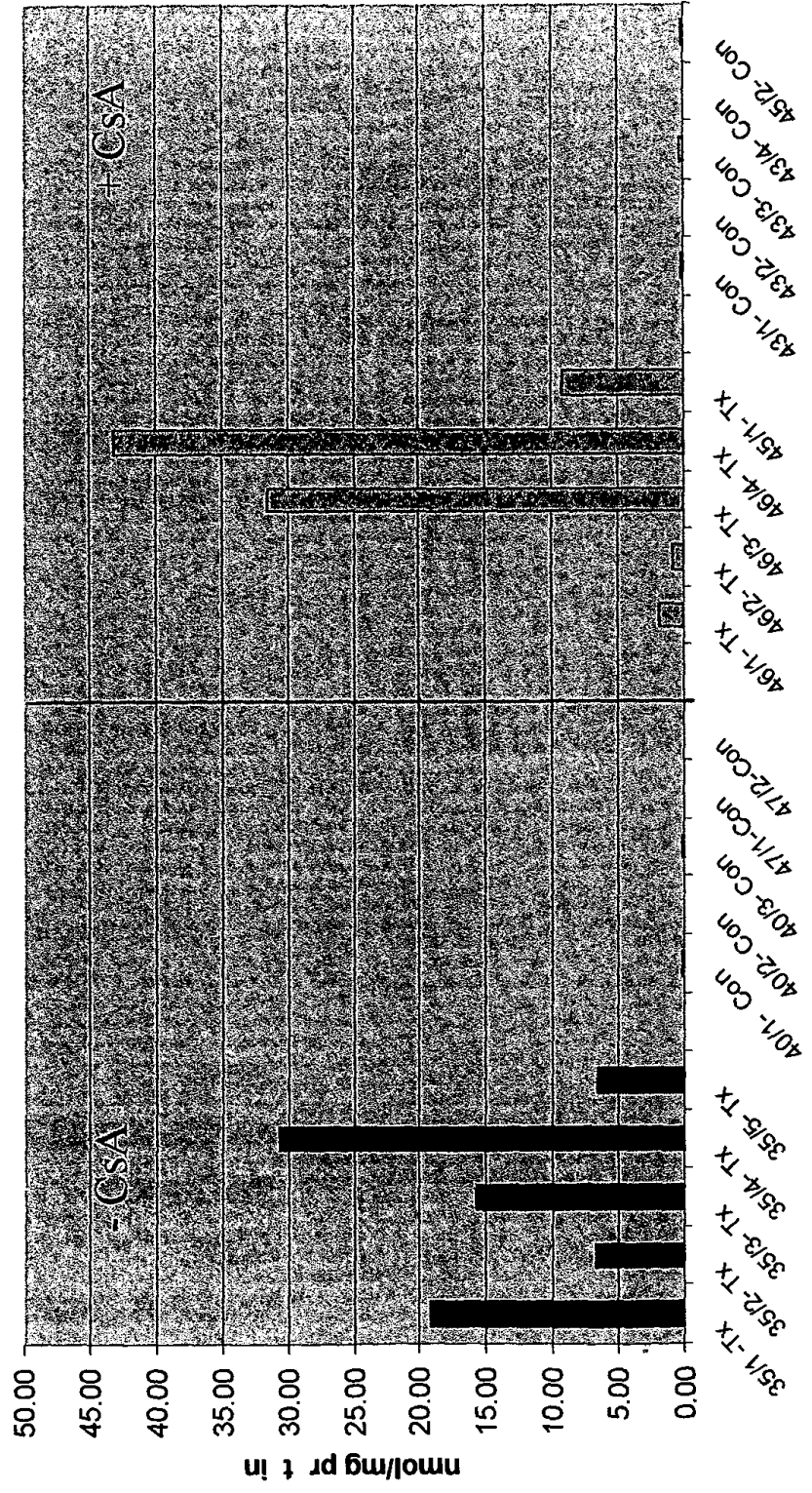
α GalA Activity in Muscles of Fabry-KO mice:
14 days after IM injection of α GalA-Transduced hMSCs



Tx = α GalA Transduced cells injected into muscle

Con = Vehicle alone injected into muscle

Figure 8b **αGalA Activity in Muscles of Fabry-KO mice:**
28 days after IM injection of αGalA-Transduced hMSCs



Tx = αGalA Transduced cells injected into muscle

Con = Vehicle alone injected into muscle

**αGala Immunostain of Liver from KO Mouse
injected IP with αGala-hMSCs on Cultispheres :
14 days**

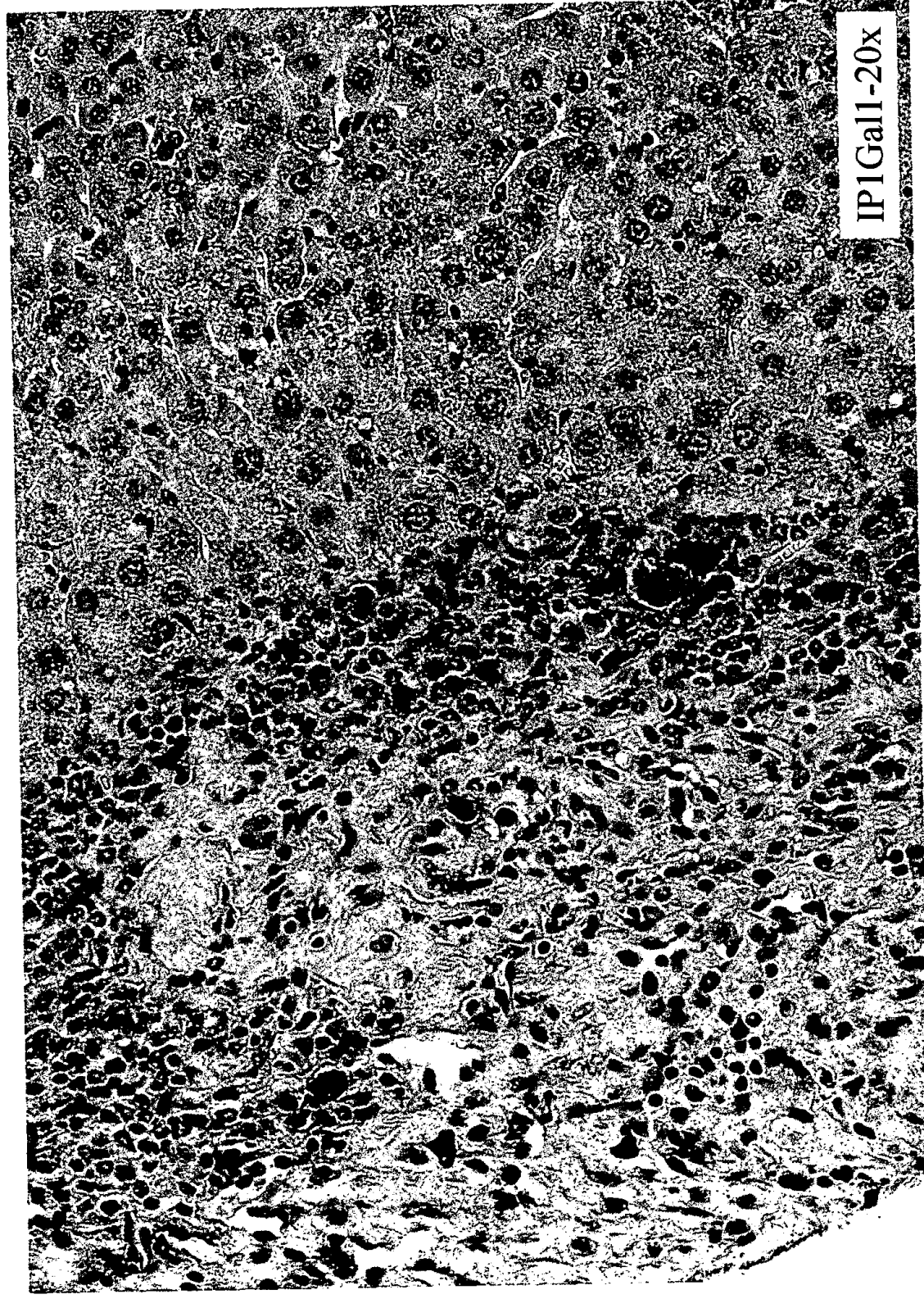


Figure-4b

α GalA Immunostain of Liver from KO Mouse
injected IP with α GalA-hMSCs on Cultispheres :
14 days



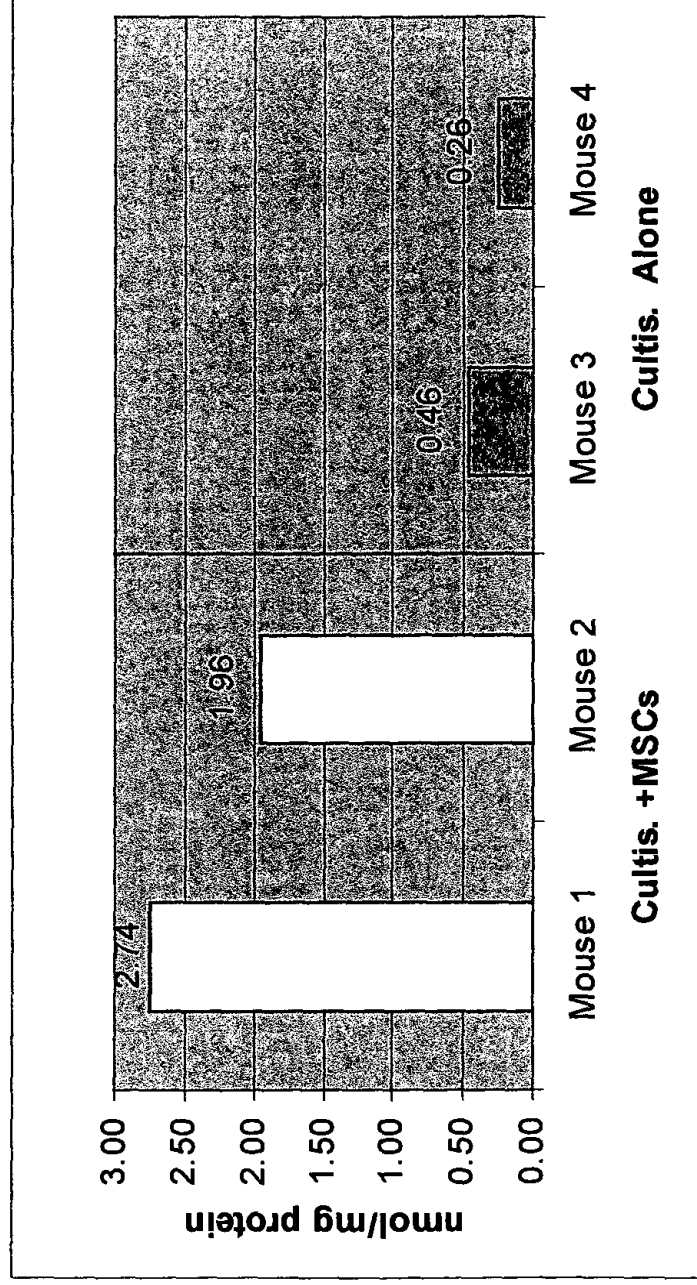
IP2Gal1a-20x

Figure 10

FOU-522660

Intra-Peritoneal Delivery of α GalA-Transduced hMSCs to Fabry Knock-Out Mice

α GalA Enzyme



Increase in α GalA is 5% of normal mouse liver activity (41 nmole/mg)